

What is Aqneursa?

Aqneursa is a prescription medicine used to treat neurologic symptoms in people with Niemann-Pick Disease Type C (NPC). It is approved for use in adults and children who weigh at least 15 kg (about 33 pounds). The non-proprietary name is levacetylleucine and also referred to as the chemical name N-acetyl-L-leucine. Aqneursa is manufactured by IntraBio.

How It Works

Aqneursa is thought to improve how cells use energy along with improving lysosome function. It may protect nerve cells and may help slow down damage caused by NPC.

Why It Matters

In clinical studies, Aqneursa improved neurologic symptoms associated with NPC, enhanced daily functioning, and showed improvements in disease progression.

How to Take Aqneursa

Form: Oral granules in single-use packets (sachets) to be suspended in a liquid

Route: By mouth, or through a feeding tube (e.g., G-tube)

How to mix: Stir the powder into about 40 mL of water, juice, or almond milk. Drink it within 30 minutes.

Food: Can be taken with or without food

Dosing Schedule

Your doctor will decide your dose based on your weight:

Weight Range	Morning Dose	Afternoon Dose	Evening Dose
15–<25 kg (33–55 lb)	1 gram	—	1 gram
25–<35 kg (55–77 lb)	1 gram	1 gram	1 gram
35 kg+ (77 lb+) and adults	2 grams	1 gram	1 gram

* Take doses about 4–6 hours apart.

What If You Miss a Dose?

- Skip the missed dose
- Take your next dose as scheduled
- Do not take two doses at once

Common Side Effects

• Stomach pain • Vomiting • Trouble swallowing • Upper respiratory infections (colds) • Headache, dizziness, tiredness • Changes in taste • Changes in liver tests (your doctor will check)

Who Should NOT Take It?

- People allergic to any ingredient in Aqneursa
- Women who are pregnant or planning to become pregnant (may harm the baby)
- Tell your doctor if you are breastfeeding

Storage & Safety Tips

- Store at room temperature
- Keep out of reach of children
- Follow your doctor's instructions
- Report any side effects

Reporting Side Effects

In addition to reporting any side effects to your healthcare providers, you are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Questions?

Always talk to your doctor or pharmacist if you have questions about your medicine.

If you ever have questions about Aqneursa - how to take it, side effects, or anything else - please call your doctor or pharmacist.

For more information see aqneursa.com